NCATS
National Center for Advancing Translational Sciences
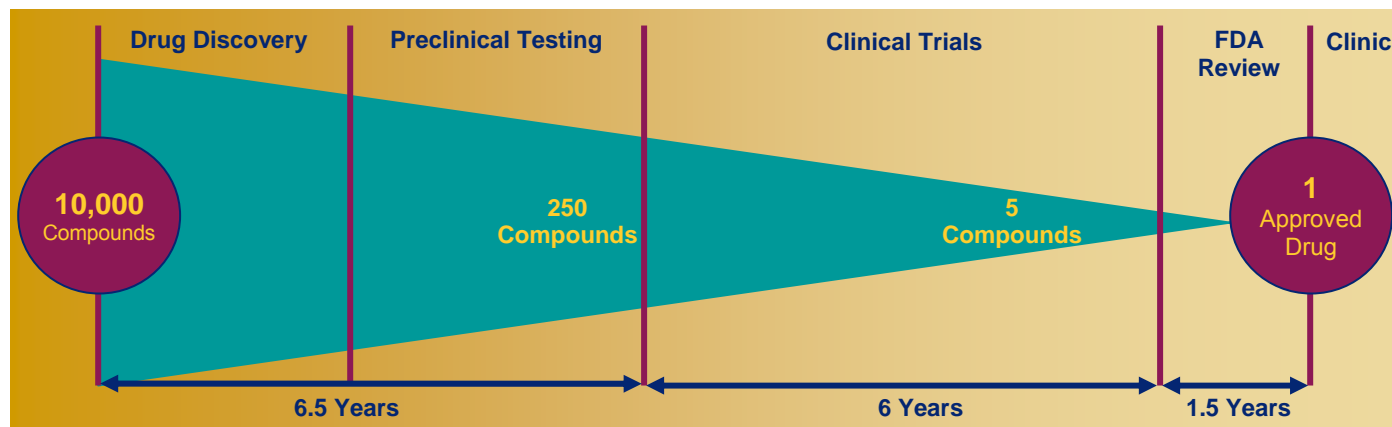*Catalyzing Innovation*

Background

Developing new diagnostics and therapeutics is a complex, costly and risk-laden endeavor. Less than one percent of compounds initially tested actually make it into a patient's medicine cabinet. NIH recognizes that the *process* for translating scientific discoveries into new tools and treatments is ripe for innovation. The National Center for Advancing Translational Sciences (NCATS) was established in FY 2012 to support rigorous research on the discipline of translation, overcoming bottlenecks to accelerate the development of diagnostics and therapeutics.

Drug Discovery	Preclinical Testing	Clinical Trials	FDA Review	Clinic
10,000 Compounds	**250** Compounds	**5** Compounds	**1** Approved Drug	
6.5 Years		**6 Years**	**1.5 Years**	

The mission of NCATS is to catalyze the generation of innovative methods and technologies that will enhance the development, testing and implementation of diagnostics and therapeutics across a wide range of human diseases and conditions. By improving the process by which diagnostics and therapeutics are developed, NCATS aims to make translational science more efficient, less expensive and less risky. In this way, NCATS complements — and does not compete with — the work of the private sector and the other NIH Institutes and Centers.

NCATS unifies programs in three areas:

► **Clinical and Translational Science Activities**
  o **Clinical and Translational Science Awards,** a national consortium of medical research institutions working to improve the way clinical and translational research is conducted nationwide

► **Rare Diseases Research and Therapeutics**
  o **Office of Rare Diseases Research,** which coordinates and supports research on rare diseases
  o **Therapeutics for Rare and Neglected Diseases,** a program to encourage and speed the development of new drugs for rare and neglected diseases

► **Re-engineering Translational Sciences**
  o **NIH Chemical Genomics Center,** which provides researchers with access to the large-scale screening and chemistry capacity necessary to identify compounds that can be used as chemical probes to validate new therapeutic targets
  o **Bridging Interventional Development Gaps,** which makes available critical preclinical resources needed for the development of new therapeutic agents
  o **Toxicology in the 21st Century,** which is screening a collection of 10,000 compounds to identify potentially toxic disruptions in biological pathways and develop ways to predict toxicity more accurately

NCATS also will develop the **Cures Acceleration Network,** which will fund a variety of initiatives designed to address scientific and technical challenges that impede transitional research.

Innovative Initiatives

Rescuing and Repurposing Drugs
Matching Drug Compounds with NIH-Funded Scientists' Ideas for New Uses

The most efficient path to new therapeutics is to find new uses for established drugs that are either already approved (drug repurposing) or unapproved (drug rescue). NCATS is working with industry to provide academic investigators and small businesses with the funding and information they need to investigate new uses for compounds from industry-provided drug collections. NCATS also is developing a comprehensive database of approved and investigational drugs and working with the Food and Drug Administration (FDA) to advance opportunities in this promising area.

Designing a Tissue Chip for Drug Screening
Mimicking Human Physiology to Predict Drug Safety and Toxicity

One of the most common reasons for the failure of new medications is toxicity that was not detected in preclinical studies. NCATS is working with the Defense Advanced Research Projects Agency and the FDA to develop a better model to predict drug safety and efficacy: a chip composed of diverse human cells and tissues that will mimic how they interact in humans. If successful, this chip could make drug safety and efficacy assessments more accurate and even make them possible earlier in the translational pipeline — enabling investigators to concentrate on the most promising new drugs.
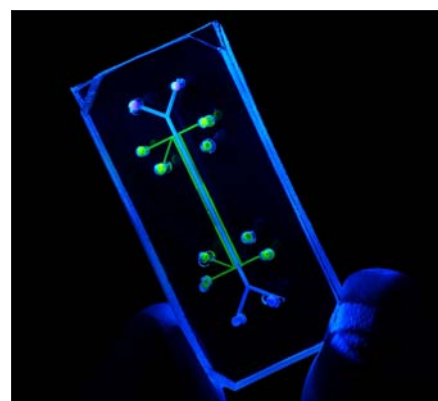

Image courtesy of the Wyss Institute.

Identifying and Validating Drug Targets
Developing New Tools and Methods to Identify Drug Targets Faster and More Efficiently

Identifying promising targets for potential new drugs, known as target validation, is a critical step in drug development, but current methods lack accuracy and reproducibility. NCATS' target validation initiative aims to develop a more strategic, harmonized and streamlined approach that incorporates new genetic and phenotypic information. In FY 2013, NCATS will collaborate with industry to develop a consortium capable of providing a repository and an analytic platform for target validation efforts. NCATS also supports pilot projects focused on discovering better approaches to validating potential new targets. This effort is a critical step in improving drug development to bring crucial therapies from the bench to the bedside.

For More Information

Visit the NCATS website at ncats.nih.gov or contact info@ncats.nih.gov.